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EL-PFDD Press Release Announcement



Prader-Willi Syndrome Association | USA to host Externally-Led Patient-Focused Drug Development Meeting to help Inform the FDA and other stakeholders on Living with Prader-Willi syndrome (PWS)

NEWS PROVIDED BY

[PWSA | USA](#)

Nov 7, 2022, 05:20 ET

The [Prader-Willi Syndrome Association | USA](#) (PWSA | USA), a non-profit organization dedicated to enhancing the quality of life and empowering those affected by Prader-Willi syndrome (PWS), announced today it will host an [externally-led patient-focused drug development](#) (EL-PFDD) meeting where the U.S. Food and Drug Administration (FDA) and other stakeholders will be in attendance. This meeting will be held in conjunction with their [PWSA | USA National Convention](#), on June 22, 2023, noon – 4:30 p.m. EST, in Orlando, Florida at the Hilton Orlando Buena Vista Palace. This historic event is intended to help educate the Agency, the public, and other stakeholders about the challenges of living with PWS and to underscore the unmet needs of the PWS community.

PWS is a variable, complex rare genetic neurobehavioral spectrum disorder with symptoms resulting from the loss of function of a critical region in the paternally derived 15th chromosome. Its symptoms vary widely in severity between individuals. Infants have failure to thrive due to feeding problems and hypotonia. Toddlers have increased weight gain. Most of the medical problems in Prader-Willi syndrome are related to obesity, hypotonia, and hypothalamic dysfunction. Other medical complications include high pain tolerance, gastrointestinal and respiratory issues, and difficulty with temperature regulation. Adrenal insufficiency occurs in a small percentage of the population. The most common and challenging symptom among those diagnosed is hyperphagia, insatiable hunger, which can lead to life-threatening obesity and is not responsive to bariatric surgery (PMID: 35416416). The worldwide average lifespan of a person with PWS is 29 years old. Currently, the only FDA-approved treatment for PWS is human growth hormone, which was approved more than 20 years ago. PWS has no cure.

“We are thrilled to host the first EL-PFDD for Prader Willi syndrome. This FDA sponsored program will allow regulators and policymakers on the Hill to hear directly from patients and caregivers about what it is like to live with and care for those who

have PWS. PWS is a rare disease that represents an incredibly high physical, emotional and economic burden on patients, their caregivers, and the healthcare system,” said Paige Rivard, CEO of PWSA | USA. “We believe that understanding the patient journey can help the FDA make more informed decisions as they review potential therapies for PWS and will assist pharmaceutical companies in the design of clinical trials for PWS patients.”

The June 22nd meeting will be offered in-person and livestreamed virtually. It will include patient testimonials, as well as remote audience participation. The meeting agenda will focus on two patient panels and audience discussion sessions on living with PWS, treatments for the disease, and potential clinical trials for PWS. It will also encourage patients and caregivers impacted by PWS to participate in a brief survey following the EL-PFDD meeting. The anonymous information gathered will be used to help guide and inform content for this meeting, the development of a meeting report that will be issued to the FDA, as well as future PWS related activities. Registration for this event and for the PWSA | USA National Convention will be available soon.

About Prader-Willi Syndrome Association | USA:

Prader-Willi Syndrome Association | USA was formed in 1975 to unite parents, professionals, and other interested citizens to enhance the quality of life of those affected by Prader-Willi syndrome. PWSA | USA empowers the PWS community through shared experiences, research, education, advocacy, and support. We can't do it alone. We invite you to help us continue to assist our loved ones affected by PWS by making a [donation](#) or learning more about how you can be involved.

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